

Adeno-Associated Virus Vectors  
Encoding Factor VIII and Methods of Using the Same

Abstract

5           The present invention provides recombinant adeno-associated virus (rAAV)  
vectors comprising a heterologous nucleotide sequence encoding factor VIII (factor  
VIII). In preferred embodiments, the factor VIII is a B-domain deleted factor VIII. Also  
provided are methods of producing a high titer stock of the inventive rAAV/factor VIII  
vectors. Another aspect of the invention is a method of delivering a nucleotide sequence  
10       encoding factor VIII to a cell, preferably for subsequent administration to a subject. The  
present invention further provides methods of administering rAAV/factor VIII to a  
subject, *e.g.*, for the treatment of hemophilia. The rAAV vector may be administered by  
any route, but is preferably administered to the liver.